Follow-up treatment with Gladskin in patients with Netherton syndrome (NS)

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Ethical review	Approved WMO
Status	Recruiting
Health condition type	Bacterial infectious disorders
Study type	Interventional

Summary

ID

NL-OMON48716

Source
ToetsingOnline

Brief title
Follow-up treatment with Gladskin in patients with Netherton syndrome

Condition

- Bacterial infectious disorders
- Skin and subcutaneous tissue disorders NEC

Synonym
ichtyosis, Netherton syndrome

Research involving
Human

Sponsors and support

Primary sponsor: Erasmus MC, Universitair Medisch Centrum Rotterdam
Source(s) of monetary or material Support: Micreos Human Health B.V.

Intervention

Keyword: Netherton syndrome (NS), Staphefekt, Staphylococcus (S.) aureus

Outcome measures

Primary outcome

1. Patient reported disease severity score using the Patient Global Assessment

(PGA)

Secondary outcome

1. Patient reported disease severity scores

- Pruritus Numerical Rating Scale (Pruritus NRS)

- Pain Numerical Rating Scale (Pain NRS)

- Desquamation Numerical Rating Scale (Desquamation NRS)

- Erythema Numerical Rating Scale (Erythema NRS)

- Sleep Numerical Rating Scale (Sleep NRS)

2. Doctor reported disease severity scores:

- Skin infection defined by the use of topical *or systemic antibiotics

- Visual index for Ichthyosis severity (VIIS)

4. S. aureus load and other microorganisms on skin and nose mucosa measured by

DNA based methods and culture.

5. Quality of life using Skindex-29 questionnaires

6. Safety and tolerability of Gladskin:

- Adverse device effect, therapy cessation for adverse device effects

- Serious Adverse Device Effect (SADE)

- Blood tests including infection parameters (leukocytes, total and

differentiation), renal function (creatinine, eGFR), liver function tests

(ASAT, ALAT, bilirubin).

Study description

Background summary

Colonization of the skin and mucosa with Staphylococcus (S.) aureus has been suggested to be associated with the multifactorial pathogenesis of NS. It might influence the skin symptoms of NS and causes recurrent skin infections in these patients. Increasing multidrug resistance of S. aureus points out the need for development of alternative treatment options next to antibiotics that are often used to treat skin symptoms and infections in NS patients. Gladskin is a product for topical use, the proprietary enzyme in the Gladskin products is called Staphefekt SA.100 (Staphefekt). Staphefekt is an endolysin that specifically lyses the cell membrane of S. aureus. In vitro results showed that Staphefekt kills S. aureus, leaving the commensal flora intact. Treatment with Gladskin might decrease S. aureus colonization of the skin and consequently decrease symptoms and severity of S. aureus related disease, such as NS.

Study objective

The aim of this study is to evaluate the effect of Gladskin treatment on the occurrence of skin symptoms in patients with NS. Secondary we want to retrieve information about the effect of Gladskin on quality of life, safety, the load of S. aureus and the further microbiome.

Study design

A descriptive case series evaluating the effects of Gladskin usage in NS patients.

Intervention

- Run in phase to monitor current treatment using medication diary (week 0-2)
- Apply Gladskin on the skin twice daily (intervention phase: week 3-10)
- Follow up phase to monitor treatment and severity scoring using a patient

diary (week 11-12)
- 3 visits of 30-60 minutes. Visit 1 is ahome visit (if patients prefer). Visit 2 and 3 are at the research department of the Erasmus MC
- Visit 1: questionnaire, medical check-up, skinswab, skinscrub, nose swab, photograph
- Visit 2 and 3: questionnaire, medical check-up, skinswab, skinscrub, nose swab, photograph, blood sampling
- Weekly short questionnaire in patient diary: medication usage, patient global assessment, sleep, pruritus, pain, erythema and desquamation NRS

Study burden and risks

The risk of using Gladskin is low. Since endolysins are proteins, they are inherently non-toxic. Theoretically toxicity can occur when in the purification process of the lysins a toxic substance of the host bacteria is co-purified. Staphefekt is tested on purity (host cell contamination and endotoxin levels) according to European regulations. Staphefekt is a large size protein molecule (>50kDa) and will not penetrate or pass intact skin. Therefore an immunogenetic reaction with the formation of antibodies against Staphefect is highly inlekely when applied on the intact skin. Literature shows that possible formed antibodies are non-neutralizing and in animal testing no adverse effects of other endolysins are seen. There is a possibility of an allergic reaction to other compnents of the product or the sampling solution (Tween 80). Because of the skin barrier impairment in NS patients, penetration of the Staphefekt protein in the skin can not be excluded. With this in mind extra safety measures will be taken:
- Patients can contact us by phone 24h a day if any problems occur
- A progressive lubrication application schedule
- Blood samples at visit 3 and 4 measuring leukocytes, creatinine, eGFR, ASAT and ALAT.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

- 18 years of age or above
- Having a diagnosis of Netherton syndrome
- Able to read patient information, fill out online questionnaires and provide informed consent

Exclusion criteria

- Systemic antibiotics within the previous 4 weeks
- Topical antibiotics within the previous 7 days
- Gladskin within the previous 7 days

Study design

Design

Study type: Interventional

Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	06-02-2019
Enrollment:	11
Type:	Actual

Medical products/devices used

Generic name:	Gladskin
Registration:	Yes - CE intended use

Ethics review

Approved WMO Date:	03-12-2018
Application type:	First submission
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	28-08-2019
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
CCMO	NL63546.078.18